

## CRISPR/Cas9 nanoparticle enabled therapy for Duchenne Muscular Dystrophy in muscle stem cells

### Grant Award Details

CRISPR/Cas9 nanoparticle enabled therapy for Duchenne Muscular Dystrophy in muscle stem cells

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-08824

**Project Objective:** CRISPR/Cas9 enabled correction of DMD in muscle stem cells

\*The awardee is pursuing two approaches to identify a therapeutic candidate. One approach aims to systemically deliver the CRISPR/Cas9 platform to correct human dystrophin gene expression in endogenous muscle stem cells in vivo (MSNP-CRISPR). The other approach aims to deliver corrected patient-derived iPSC-derived human skeletal muscle progenitor cells (SMPCs) to repopulate the endogenous stem cell niche in combination with in vivo delivery of chemoattractants by nanoparticles that are released in the muscle to facilitate SMPC homing to the site (MSNP-STEM).

**Investigator:**

<b>Name:</b>	April Pyle
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

**Disease Focus:** Muscular Dystrophy, Skeletal/Smooth Muscle disorders

**Human Stem Cell Use:** iPS Cell

**Award Value:** \$2,150,400

**Status:** Active

### Grant Application Details

**Application Title:** CRISPR/Cas9 nanoparticle enabled therapy for Duchenne Muscular Dystrophy in muscle stem cells

**Public Abstract:****Research Objective**

Gene correction of muscle stem cells

**Impact**

These studies will develop a gene editing based therapy for one of the most prevalent lethal childhood disorders called Duchenne Muscular Dystrophy.

**Major Proposed Activities**

- To identify the best MSNP-CRISPR candidates for CRISPR/Cas9 plasmid delivery in vitro to muscle stem cells
- To identify the best MSNP-STEM candidates suitable for delivery of the optimal chemo attractant that enables stem cell migration in vitro
- To identify the optimal MSNP-CRISPR and MSNP-STEM candidates from biodistribution studies after systemic injection
- To determine the efficiency of MSNP-CRISPR and MSNP-STEM approaches for delivering CRISPR/Cas9 platform to the stem cell niche.
- To identify the MSNP delivery strategy that results in restoration of functional dystrophin protein and improved muscle strength after long-term satellite cell correction or reconstitution.

**Statement of Benefit to California:**

Duchenne Muscular Dystrophy is a progressive muscle wasting disorder with life expectancy of approximately age 20 with incidence of 1 in 5,000 live male births. Because it is a chronic disorder, this disease is devastating to families, involves extensive medical expenses and loss of employment for caregivers. School-age children require a classroom aid and an IEP. A treatment for DMD could reduce health care costs, time lost from work and burden on the public school system.

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